Real World Evidence

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Applying Advanced Data Privacy Methods to Real World Data (RWD)

Scope

The goal of this project is to develop a comprehensive, cross-disciplinary resource to support individuals and organisations in applying advanced privacy-preserving techniques to real-world data (RWD). Given the evolving landscape of privacy regulations and the increasing complexity of data sources - including integrating machine learning (ML), artificial intelligence (AI) and large language models (LLMs) into healthcare pipelines - this project will serve as a critical foundation for advancing privacy-preserving data integration frameworks for RWD.

Proposed End Date

Q4 2026

Deliverable Type

White paper/ guideline

Leads

Elena Valkanova

Key Achievements This Quarter:

- Kick-off meeting: 08 October 2025
- Meeting: 12 November 2025

Objectives:

- Review the current tracker and workstream structure.
- Select Co-Leads and workstream assignments.
- Discuss term deliverables and action plans - part of the development of the white paper/guideline and supporting materials.
- Discuss the submission of the PHUSE US Connect 2026 RWE Catalyst Challenge: Theme: Transforming Real-World Evidence with NLP & AI.

Deliverables & Targets Planned for the Next Quarter:

- Workstream 1-2: Review supplementary files (e.g. open data sources, method libraries). Develop a structured plan to test and benchmark existing privacy-preserving methods (DP, FL, synthetic generation) on open RWD sources.
- Workstream 3-4: Review shared reference materials. Draft the introduction and the outline of the white paper/guideline, defining the scope and proposed sections for governance, compliance and adoption.

Project Status

Project Status: Green
Accepting New Members – *with programming experience with RWD.*

- On track – review of existing RWD/RWE frameworks, guidelines, templates relating to data privacy.

Estimands for RWD/RWE

Scope

The focus of this project is on best practices of RWD estimands, not on their implementation using data standards. The team has representation from the PHUSE Implementation of Estimands (ICH E9 (R1)) using Data Standards project team, which has a white paper nearing publication, and the Estimands Implementation Working Group (EIWG) sub team on HTA and RWE, both with estimands expertise, along with representation from teams under the RWE Working Group. The EIWG sub team on HTA and RWE started in late 2022, and has a cross-functional membership, including regulators, statisticians, epidemiologists and health economists.

Q2 2027

Proposed End Date

White paper & webinar series

Deliverable Type

Matt Baldwin, Ksenia Titorenko & Paramita Chakraborty

Leads

White paper stable draft key sections review

Key Achievements This Quarter:

- Draft white paper key sections for internal review
- Third webinar
- Planning for the US Connect 2026 RWE Pavilion workshop

Deliverables & Targets Planned for the Next Quarter:

Project Status: Green

- In progress

Project Status

Missing Data Imputation in RWD Exploration of Multiple Techniques on Open-Source Data

Scope

We aim to publish a white paper which will explore multiple models available for missing data imputation, and share with the wider group the potential of each model and its efficiency in dealing with different kinds of missing data. The model efficiency will be compared using a single open-source simulated dataset.

Q4 2026

Proposed End Date

White paper

Deliverable Type

Likhita Kolli, Sujith Mididoddi & Tuhin James Paul

Leads

- Successfully planned the PHUSE US Hackathon. Registrations are now live on the PHUSE website.
- Reviewed and analysed multiple data sources to assess their suitability for supporting imputation. MIMIC-III and NACC datasets have been evaluated, and we have now moved to the UC Berkeley dataset shared by Elena.

Key Achievements This Quarter:

- Introduce controlled missingness in one selected dataset once finalised by the team.
- Set up a dedicated GitHub repository for the missing data imputation work.
- Convert the existing literature review into an AI-assisted rapid review.
- Finalise and submit the agreed-upon blog topics by the end of the quarter.

Deliverables & Targets Planned for the Next Quarter:

Project Status: Green
Accepting New Members

- Dataset identification and suitability assessment are underway.
- Literature review approach has been revised to an AI-assisted rapid review due to limited attendance.
- PHUSE US Hackathon preparations are progressing very well.

Project Status

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Quality and Reusability of Real World Data

Scope

Real-world data sources have been used for many years in the pharmaceutical industry. They are useful for validating research questions and protocol set-up, patient and centre selection, post-registry safety follow-up and market access. However, the use for regulatory submissions is new. For this, the requirements are more stringent, and we need a robust process to assess quality, accuracy, appropriateness of the data and compliance to regulatory requirements. How are we going to set ourselves up for success? We shall focus on:

- Selecting the appropriate data source
- Ensuring the data sources are accurate and traceable
- How we assess the quality of the data.

Proposed End Date

Q1 2026

Deliverable Type

- Guideline review blog posts
- Data vendor feasibility blog posts
- White paper

Key Achievements This Quarter:

Final paper for public review in latest stage

Deliverables & Targets Planned for the Next Quarter:

- Final paper for public review
- Paper submission for journal

Leads

Berber Snoeijer & Ashwin Rai

Project Status

Project Status: Green

- Final in progress

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RWD Guideline for Programming and Analysis Processes

Scope

The project will bring together statistical programmers across pharma to collaborate and provide their input to create an RWE guideline/white paper for the industry.

Proposed End Date

Q4 2025

Deliverable Type

White paper

Leads

Dhruba Sikdar

Key Achievements This Quarter:

Paper consolidation

Deliverables & Targets Planned for the Next Quarter:

Final paper for publication

Project Status

Project Status: Green

- Almost final

Submitting Real World Data

Scope

The scope of the project includes developing collaterals for submission of real-world data through mutual discussion, knowledge, and experience sharing by industry colleagues. The collaterals will be developed by undertaking research of draft guidance documents on real-world evidence by regulatory bodies, and by analysing members' lessons learnt from completed case studies. The scope of this project will include submission of real-world data obtained from primary and widely used real-world data sources. These may include key electronic health record sources, patient reported outcomes, widely used and accepted claims data sources, and other commonly used observational data. This project will also touch upon the data curation and ingestion processes pertaining to clinical operational as well as submission data.

Q4 2025

Proposed End Date

White paper

Deliverable Type

Parag Shiralkar

Leads

Key Achievements This Quarter:

- Review of DRG document
- Alignment with the ODS Working Group and preparation for the US Connect 2026 RWE Pavilion workshop

Deliverables & Targets Planned for the Next Quarter:

Input and preparations for the US Connect 2026 RWE Pavilion workshop

Project Status: Green

- Wrapping up

Project Status

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Using OMOP and Other Real World Data Standards to Support Regulatory Submissions

Scope

The scope of the project includes developing awareness of data standards specific to real-world data to support regulatory purposes. Common data models (CDMs) and taxonomies that are specific to the most commonly used real-world data sources by industry, member companies and regulatory bodies across the globe will be in scope, such as electronic health record sources, patient reported outcomes, widely accepted claims data sources, and other commonly used observational data. OMOP and the OHDSI community will be the focus and will be evaluated against other CDMs and standards (such as PCORnet, Sentinel and CDISC).

Q4 2026

Proposed End Date

White paper

Deliverable Type

Berber Snoeijer, Alexa Parliyan & Sanket Kalyankar

Leads

First draft chapters of white paper

Key Achievements This Quarter:

More chapters of white paper, preparation for the US Connect 2026 RWE Pavilion workshop

Deliverables & Targets Planned for the Next Quarter:

Project Status: Green
Accepting New Members

- Draft in progress

Project Status

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